

IN THE UNITED STATES PATENT AND TRADEMARK OFFICE

In re Patent Application of
Krzysztof MASTERNAK et al.
Application No.: 09/840,243
Filed: April 24, 2001



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Group Art Unit: 1623
Examiner: Unassigned

For: NEW TRANSCRIPTION FACTOR OF
MHC CLASS II GENES, SUBSTANCES
CAPABLE OF INHIBITING THIS NEW
TRANSCRIPTION FACTOR AND
MEDICAL USES OF THESE
SUBSTANCES

INFORMATION DISCLOSURE STATEMENT
TRANSMITTAL LETTER

Assistant Commissioner for Patents
Washington, D.C. 20231

Sir:

Enclosed is an Information Disclosure Statement and accompanying form PTO-1449 for the above-identified patent application.

- ☒ [X] No additional fee for submission of an IDS is required.
- ☐ [] The fee of \$180.00 (126) as set forth in 37 C.F.R. § 1.17(p) is also enclosed.
- ☐ [] A certification under 37 C.F.R. § 1.97(e) is also enclosed.
- ☐ [] A certification under 37 C.F.R. § 1.97(e), and the fee of \$180.00 (126) as set forth in 37 C.F.R. § 1.17(p) are also enclosed.
- ☐ [] Charge \$_____ to Deposit Account No. 02-4800 for the fee due.
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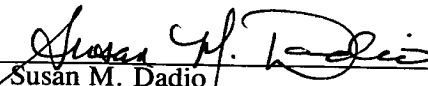
The Commissioner is hereby authorized to charge any appropriate fees under 37 C.F.R. §§ 1.16, 1.17 and 1.21 that may be required by this paper, and to credit any overpayment, to Deposit Account No. 02-4800. This paper is submitted in duplicate.

Respectfully submitted,

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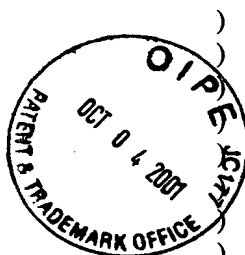
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INFORMATION DISCLOSURE STATEMENT

Assistant Commissioner for Patents
Washington, D.C. 20231

Sir:

In accordance with the duty of disclosure as set forth in 37 C.F.R. § 1.56, Applicants hereby submit the following information in conformance with 37 C.F.R. §§ 1.97 and 1.98.

Pursuant to 37 C.F.R. § 1.98, a copy of each of the documents cited is enclosed.

OTHER DOCUMENTS

1. KARLSSON, "Separation Efficiency of Slurry-Packed Liquid Chromatography Microcolumns with Very Small Inner Diameters", *Anal. Chem.*, 1988, pp. 1662-65, vol. 60, American Chemical Society, Washington, D.C.
2. YATES et al., "Method to Correlate Tandem Mass Spectra of Modified Peptides to Amino Acid Sequences in the Protein Database", *Anal. Chem.*, 1995, pp. 1426-36, vol. 67, American Chemical Society, Washington, D.C.
3. ZHOU et al., "Human MHC Class II Gene Transcription Directed by the Carboxyl Terminus of CIITA, One of the Defective Genes in

- Type II MHC Combined Immune Deficiency", *Immunity*, pp. 545-553, vol. 2, Cell Press, Philadelphia, PA.
4. FUERST et al., "Eukaryotic transient-expression system based on recombinant vaccinia virus that synthesizes bacteriophage T7 RNA polymerase", *Proc. Natl. Acad. Sci.*, 1986, pp. 8122-26, vol. 83, National Academy of Sciences, Washington, D.C.
 5. BAEUERIE et al., "NF- κ B: Ten Years After - Meeting Review", *Cell*, 1996, pp. 13-20, vol. 87, Cell Press, Philadelphia, PA.
 6. ALKHATIB et al., "Cloning and expression of cDNA for human poly(ADP-ribose) polymerase", *Proc. Natl. Acad. Sci.*, 1987, pp. 1224-28, vol. 84, National Academy of Sciences, Washington, D.C.
 7. STEIMLE et al., "Complementation Cloning of an MHC Class II Transactivator Mutated in Hereditary MHC Class II Deficiency (or Bare Lymphocyte Syndrome)", *Cell*, 1993, pp. 135-46, vol. 75, Cell Press, Philadelphia, PA.
 8. RILEY et al., "Activation of Class II MHC Genes Requires Both the X Box Region and the Class II Transactivator (CIITA)", *Immunity*, 1995, pp. 533-43, vol. 2, Cell Press, Philadelphia, PA.
 9. LISOWSKA-GROSPIERRE, et al., "Two complementation groups account for most cases of inherited MHC class II deficiency", *Human Molecular Genetics*, 1994, pp. 953-58, vol. 3, Oxford University Press, Oxford, UK.
 10. BATCHELOR et al., "The Structure of GABP α/β : An ETS Domain - Ankyrin Repeat Heterodimer Bound to DNA", *Science*, 1998, pp. 1037-1041, vol. 279, American Association for the Advancement of Science, Washington, D.C.
 11. BENICHOU et al., "Class II-antigen-negative patient and mutant B-cell lines represent at least three, and probably four, distinct genetic defects defined by complementation analysis", *Proc. Natl. Acad.*

- Sci.*, 1991, pp. 4285-88, vol. 88, National Academy of Sciences, Washington, D.C.
12. BORK, "Hundreds of Ankyrin-Like Repeats in Functionally Diverse Proteins: Mobile Modules That Cross Phyla Horizontally", *PROTEINS: Structure, Function, and Genetics*, 1993, pp. 363-74, vol 17, John Wiley & Sons, New York, New York.
 13. DURAND, "Functional Complementation of Major Histocompatibility Complex Class II Regulatory Mutants by the Purified X-Box-Binding Protein RFX", *Molecular and Cellular Biology*, 1994, pp. 6839-47, vol. 14(10), American Society for Microbiology, Washington, D.C.
 14. SANCHEZ et al., "The DNA-Binding Defect Observed in Major Histocompatibility Complex Class II Regulatory Mutants Concerns Only One Member of a Family of Complexes Binding to the X Boxes of Class II Promoters", *Molecular and Cellular Biology*, 1992, pp. 4076-83, vol. 12(9), American Society for Microbiology, Washington, D.C.
 15. KRETISOVALI et al., "Involvement of CREB Binding Protein in Expression of Major Histocompatibility Complex Class II Genes via Interaction with the Class II Transactivator", *Molecular and Cellular Biology*, 1998, pp. 6777-83, vol. 18(11), American Society for Microbiology, Washington, D.C.
 16. WATANABE et al., "cDNA Cloning of Transcription Factor E4TF1 Subunits with Ets and Notch Motifs", *Molecular and Cellular Biology*, 1993, pp. 1385-91, vol. 13(3), American Society for Microbiology, Washington, D.C.
 17. VILLARD et al., "Mutation of RFXAP, A regulator of MHC Class II Genes, In Primary MHC Class II Deficiency", *The New England Journal of Medicine*, 1997, pp. 748-53, vol. 337, Massachusetts Medical Society, Boston, MA.

18. BONTRON et al., "Two novel mutations in the MHC class II transactivator CIITA in a second patient from MHC class II deficiency complementation group A", *Human Genet.*, 1997, pp. 541-46, vol. 99, Springer-Verlag, London, England.
19. VAN DEN ELSEN et al., "Regulation of MHC class I and II gene transcription: differences and similarities", *Immunogenetics*, 1998, pp. 208-21, vol. 48, Springer-Verlag, London, England.
20. TSANG et al., "Mutational Analysis of the DRA Promoter: cis-Acting Sequences and trans-Acting Factors", *Molecular and Cellular Biology*, 1990, pp. 711-19, vol. 10(2), American Society for Microbiology, Washington, D.C.
21. STEIMLE et al., "A novel-DNA-binding regulatory factor is mutated in primary MHC class II deficiency (bare lymphocyte syndrome)", *Genes & Development*, 1995, pp. 1021-32, vol. 9, Cold Spring Harbor Laboratory Press, Woodbury, New York.
22. ACCOLLA, "Human B Cell Variants Immunoselected Against A Single Ia Antigen Subset Have Lost Expression of Several Ia Antigen Subsets", *J. Exp. Med.*, 1983, pp. 1053-58, vol. 157, The Rockefeller University Press, New York, New York.
23. BALDWIN, "The NF- κ B and I κ B Proteins: New Discoveries and Insights", *Annu. Rev. Immunol.*, 1996, pp. 649-81, vol. 14, Annual Reviews, Palo Alto, CA.
24. BASTA et al., "Identification of An Interferon- γ Response Region 5' of the Human Histocompatibility Leukocyte Antigen DR α Chain Gene Which is Active in Human Glioblastoma Multiforme Lines", *The Journal of Immunology*, 1987, pp. 1275-80, vol. 138, American Association of Immunologists, Bethesda, Maryland.
25. BONTRON et al., "Efficient Repression of Endogenous Major Histocompatibility Complex Class II Expression through Dominant Negative CIITA Mutants Isolated by a Functional Selection

- Strategy", *Molecular and Cellular Biology*, 1997, pp. 4249-58, vol. 17(8), American Society for Microbiology, Washington, D.C.
26. DURAND et al., "RFXAP, a novel subunit of the RFX DNA binding complex is mutated in MHC class II deficiency", *The EMBO Journal*, 1997, pp. 1045-55, vol. 16(5), Oxford University Press, Oxford, UK.
27. EMERY et al., "RFX proteins, a novel family of DNA binding proteins concerved in the eukaryotic kingdom", *Nucleic Acids Research*, 1996, pp. 803-07, vol. 24(5), Oxford University Press, Oxford, UK.
28. KLEIN et al., "Bone Marrow Transplantation in Major Histocompatibility Complex Class II Deficiency: A Single-Center Study of 19 Patients", *Blood*, 1995, pp. 580-87, vol. 85(2), The American Society of Hematology, Washington, D.C.
29. CLAUSEN et al., "Residual MHC Class II Expression on Mature Dendritic Cells and Activated B Cells in RFX5-Deficient Mice", *Immunity*, 1998, pp. 143-55, vol. 8, Cell Press, Philadelphia, PA.
30. CHANG et al., "Mice Lacking the MHC Class II Transactivator (CIITA) Show Tissue-Specific Impairment of MHC Class II Expression", *Immunity*, 1996, pp. 167-78, vol. 4, Cell Press, Philadelphia, PA.
31. HASEGAWA et al., "Two B cell factors bind the HLA-DRA X box region and recognize different subsets of HLA class II promoters", *Nucleic Acids Research*, 1991, pp. 6269-76, vol. 19(22), Oxford University Press, Oxford, UK.
32. IWABUCHI, et al., "Two cellular proteins that bind to wold-type but not mutant p53", *Proc. Natl. Acad. Sci.*, 1994, pp. 6098-6102, vol. 91, National Academy of Sciences, Washington, D.C.
33. REITH et al., "Function of major histocompatibility complex class II promoters requires cooperative binding between factors RFX and

- NF-Y", *Proc. Natl. Acad. Sci.*, 1994, pp. 554-58, vol. 91, National Academy of Sciences, Washington, D.C.
34. REITH et al. "MHC class II regulatory factor RFX has a novel DNA-binding domain and a functionally independent dimerization domain", *Genes & Development*, 1990, pp. 1528-40, vol. 4, Cold Spring Harbor Laboratory Press, Woodbury, New York.
35. DE PRÉVAL et al., "A trans-acting class II regulatory gene unlinked to the MHC controls expression of HLA class II genes", *Nature*, 1985, pp. 291-293, vol. 318, Nature Publishing Group, England.
36. WILM et al., "Femtomole sequencing of proteins from polyacrylamide gels by nano-electrospray mass spectrometry", *Nature*, 1996, pp. 466-69, vol. 379, Nature Publishing Group, England.
37. MASTERNAK et al., "A gene encoding a novel RFX-associated transactivator is mutated in the majority of MHC class II deficiency patients", *Nature Genetics*, 1998, pp. 273-77, vol. 20, Nature Publishing Group, England.
38. MCDONALD et al., "Ankyrin for clues about the function of p16INK4a", *Nature Structural Biology*, 1998, pp. 85-88, vol. 5(2), Nature Publishing Group, England.
39. MERIKA et al., "Recruitment of CBP/p300 by the IFN β Enhanceosome is Required for Synergistic Activation of Transcription", *Molecular Cell*, 1998, pp. 277-87, vol. 1, Cell Press, Philadelphia, PA.
40. MAHANTA et al., "Transactivation by CIITA, the type II bare lymphocyte syndrome-associated factor, requires participation of multiple regions of the TATA box binding protein", *Proc. Natl. Acad. Sci.*, 1997, pp. 6324-29, vol. 94, National Academy of Sciences, Washington, D.C.

41. MACH et al., "Regulation of MHC Class II Genes: Lessons from a Disease", *Annu. Rev. Immunol.*, 1996, pp. 301-31, vol. 14, Annual Reviews, Inc., Palo Alto, CA.
42. BENNETT, "Ankyrins", *The Journal of Biological Chemistry*, 1992, pp. 8703-06, vol. 267(13), John Wiley & Sons, New York, New York.
43. MORENO et al., "Regulatory Factor X, a Bare Lymphocyte Syndrome Transcription Factor, Is a Multimeric Phosphoprotein Complex", *The Journal of Immunology*, 1997, pp. 5841-48, vol. 158, American Association of Immunologists, Bethesda, Maryland.
44. SEIDL et al., "Genetic Complexity of Regulatory Mutants Defective for HLA Class II Gene Expression", *The Journal of Immunology*, 1992, pp. 1576-84, vol. 148, American Association of Immunologists, Bethesda, Maryland.
45. LOUIS-PLENCE, "Formation of a Regulatory Factor X/X2 Box-Binding Protein/Nuclear Factor-Y Multiprotein Complex on the Conserved Regulatory Regions of HLA Class II Genes", *The Journal of Immunology*, 1997, pp. 3899-3909, vol. 159, American Association of Immunologists, Bethesda, Maryland.
46. CASPER et al., "Successful treatment with an unrelated-donor bone marrow transplant in an HLA-deficient patient with severe combined immune deficiency ("bare lymphocyte syndrome")", *J. Pediatr.*, 1990, pp. 262-5, vol. 116, C.V. Mosby Co., St. Louis, Missouri.
47. TOURAINE et al., "Combined immunodeficiency disease associated with absence of cell-surface HLA-A and -B antigens", *The Journal of Pediatrics*, 1978, pp. 47-51, vol. 93(1), C.V. Mosby Co., St. Louis, Missouri.
48. THOMPSON et al., "Convergence of Ets- and Notch-Related Structural Motifs in a Heteromeric DNA Binding Complex",

- Science*, 1991, pp. 762-68, vol. 253, American Association for the Advancement of Science, Washington, D.C.
49. STEIMLE et al., "Regulation of MHC Class II Expression by Interferon- γ Mediated by the Transactivator Gene CIITA", *Science*, 1994, pp. 106-09, vol. 265, American Association for the Advancement of Science, Washington, D.C.
50. ROEDER, "Role of General and Gene-specific Cofactors in the Regulation of Eukaryotic Transcription", *Cold Spring Harbor Symposia on Quantitative Biology*, 1998, pp. 201-18, vol. LXIII, Cold Spring Harbor Laboratory Press, Woodbury, New York.
51. OCHS et al., "Molecular Basis of Major Histocompatibility Complex Class II Deficiency", *Primary Immunodeficiency Diseases - A Molecular and Genetic Approach*, 1999, pp. 167-80, Oxford University Press, Inc., New York, New York.
52. REITH et al., "Cooperative Binding between Factors RFX and X2bp to the X and X2 Boxes of MHC Class II Promoters", *The Journal of Biological Chemistry*, 1994, pp. 20020-25, vol. 269(31), John Wiley & Sons, New York, New York.
53. OTTEN et al., "Quantitative control of MHC class II expression by the transactivator CIITA" *Eur. J. Immunol.*, 1998, pp. 473-78, vol. 28, Wiley-VCH Verlag GmbH, Weinheim, Germany.
54. NAGARAJAN et al., "RFX-B Is The Gene Responsible for the Most Common Cause of the Bare Lymphocyte Syndrome, an MHC Class II Immunodeficiency", *Immunity*, 1999, pp. 153-62, vol. 10, Cell Press, Philadelphia, PA.
55. MORENO et al., "CREB Regulates MHC Class II Expression in a CIITA-Dependent Manner", *Immunity*, 1999, pp. 143-51, vol. 10, Cell Press, Philadelphia, PA.
56. KLEIN et al., "Major histocompatibility complex class II deficiency: Clinical manifestations, immunologic features, and

- outcome", *J. Pediatr.*, 1993, pp. 921-8, vol. 123, Mosby-Year Book, Inc., St. Louis, Missouri.
57. LISOWSKA-GROSPIERRE et al., "A Defect in the Regulation of Major Histocompatibility Complex Class II Gene Expression in Human HLA-DR Negative Lymphocytes from Patients with Combined Immunodeficiency Syndrome", *J. Clin. Invest.*, 1985, pp. 381-85, vol. 76, The Journal of Clinical Investigation, Ann Arbor, Michigan.
58. WU et al., "Transcription: Common cofactors and cooperative recruitment", *Current Biology*, 1999, pp. R606-609, vol. 9, Cell Press, Philadelphia, PA.
59. SATOLA et al., "Congenital Immunodeficiency with a Regulatory Defect in MHC Class II Gene Expression Lacks a Specific HLA-DR Promoter Binding Protein, RF-X", *Cell*, 1988, pp. 897-906, vol. 53, Cell Press, Philadelphia, PA.
60. BOSS, "Regulation of transcription of MHC class II genes", *Current Opinion in Immunology*, 1997, pp. 107-13, vol. 9, Cell Press, Philadelphia, PA.
61. CAREY, "The Enhanceosome and Transcriptional Synergy", *Cell*, 1998, pp. 5-8, vol. 92, Cell Press, Philadelphia, PA.
62. LAMARCO et al., "Identification of Ets- and Notch-Related Subunits in GA Binding Protein", *Science*, 1991, pp. 789-92, vol. 253, American Association for the Advancement of Science, Washington, D.C.
63. WRIGHT et al., "CIITA stimulation of transcription factor binding to major histocompatibility complex class II and associated promoters in vivo", *Proc. Natl. Acad. Sci.*, 1998, pp. 6267-72, vol. 95, National Academy of Sciences, Washington, D.C.

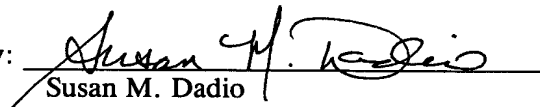
64. THANOS et al., "Virus Induction of Human IFN β Gene Expression Requires the Assembly of an Enhanceosome", *Cell*, 1995, pp. 1091-1100, vol. 83, Cell Press, Philadelphia, PA.
65. VAN HUIJSDUIJNEN et al., "Co-evolution from yeast to mouse: cDNA cloning of the two NF-Y (CP-1/CBF) subunits", *The EMBO Journal*, 1990, pp. 3119-27, vol. 9(10), Oxford University Press, Oxford, UK.
66. MUHLETHALER-MOTTET et al., "Expression of MHC class II molecules in different cellular and functional compartments is controlled by differential usage of multiple promoters of the transactivator CIITA", *The EMBO Journal*, 1997, pp. 2851-60, vol. 16(10), Oxford University Press, Oxford UK.
67. SHAPIRO et al., "Laboratory Methods - A High-Efficiency HeLa Cell Nuclear Transcription Extract", *DNA*, 1988, pp. 47-55, vol. 7, Mary Ann Lieber, Inc., Larchmont, New York.
68. ENG et al., "An approach to correlate Tandem Mass Spectral Data of peptides with amino acid sequences in a protein database" *Journal of American Society of Mass Spectrom.*, 1994, pp. 976-89, vol. 5, Elsevier, England.
69. HUME et al., "Bare Lymphocyte Syndrome: Altered HLA Class II Expression in B Cell Lines Derived from Two Patients", *Human Immunology*, 1989, pp. 1-11, vol. 25, Elsevier, England.
70. GRISCELLI et al., "Combined Immunodeficiency with Defective Expression in MHC Class II Genes", *Immunodeficiency Reviews*, 1989, pp. 141-54, vol. 2, chapter 8, England.
71. LATCHMAN, "Transcription-Factor Mutations and Disease", *The New England Journal of Medicine*, 1996, pp. 28-33, vol. 334(1), Massachusetts Medical Society, Boston, MA.

The documents are being submitted within 3 months of the filing or entry of the national stage of this application or before the first Office Action on the merits, whichever is later, therefore no fee or certification is required under 37 C.F.R. § 1.97(b).

To assist the Examiner, the documents are listed on the attached form PTO-1449. It is respectfully requested that an Examiner initialed copy of this form be returned to the undersigned.

Respectfully submitted,

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